#### (12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

#### (19) World Intellectual Property Organization International Bureau



## - 1 (0.11) 2 (0.11) 1 (0.11) 1 (0.11) 1 (0.11) 1 (0.11) 1 (0.11) 1 (0.11) 1 (0.11) 1 (0.11) 1 (0.11) 1 (0.11)

## (43) International Publication Date 14 October 2004 (14.10.2004)

#### PCT

# (10) International Publication Number WO 2004/087069 A2

(51) International Patent Classification7:

**A61K** 

(21) International Application Number:

PCT/US2004/009572

- (22) International Filing Date: 26 March 2004 (26.03.2004)
- (25) Filing Language:

English

(26) Publication Language:

English

(30) Priority Data:

60/458,079

27 March 2003 (27.03.2003) U.

- (71) Applicant (for all designated States except US): PTC THERAPEUTICS, INC. [US/US]; 100 Corporate Court, Middlesex Business Center, South Plainfield, NJ 07080 (US).
- (72) Inventor; and

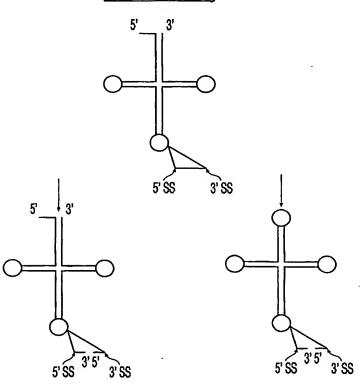
(75) Inventor/Applicant (for US only): TROTTA, Christopher, R. [US/US]; 198 Scobee Lane, Somerset, NJ 08873 (US).

- (74) Agents: CORUZZI, Laura, A. et al.; Jones Day, 222 East 41st Street, New York, NY 10017-6702 (US).
- (81) Designated States (unless otherwise indicated, for every kind of national protection available): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BW, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NA, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (84) Designated States (unless otherwise indicated, for every kind of regional protection available): ARIPO (BW, GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PL, PT, RO, SE, SI, SK,

[Continued on next page]

(54) Title: METHODS OF IDENTIFYING COMPOUNDS THAT TARGET tRNA SPLICING ENDONUCLEASE AND USES OF SAID COMPOUNDS AS ANTI-PROLIFERATIVE AGENTS

#### HTS Fluorescent Screening



(57) Abstract: The present invention relates to a method for screening and identifying compounds that modulate the activity tRNA splicing endonuclease. In particular, the invention provides assays for the identification of compounds that inhibit animalia tRNA splicing endonuclease. The methods of the present invention provide a simple, sensitive assay for high-throughput screening of libraries of compounds to identify pharmaceutical leads useful for treating and/or preventing cancer.

### WO 2004/087069 A2



TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW,

ML, MR, NE, SN, TD, TG).

#### Published:

without international search report and to be republished upon receipt of that report

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.